

## Minoryx Therapeutics completes phase 1 clinical trial for MIN-102

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**MIN-102 was well-tolerated at much higher doses than those required for efficacy**



Minoryx Therapeutics, a drug development company specialized in the discovery of new drugs for orphan diseases, announced that it has successfully completed its phase 1 trial with MIN-102.

MIN-102 targets X-linked adrenoleukodystrophy (X-ALD), a rare and chronically debilitating life threatening neurodegenerative disease. There are two main clinical phenotypes of X-ALD: adrenomyeloneuropathy (AMN), characterized by progressive motor dysfunction, and inflammatory cerebral ALD (cALD), characterized by severe neuroinflammation leading to early death. There are currently no pharmacological treatments for X-ALD. MIN-102 is the only product in development for potential use across all the main phenotypes.

The phase 1 trial was a combined single- and multiple-ascending dose study with the aim of assessing pharmacokinetics, safety and tolerability of MIN-102 in healthy male volunteers. Additionally, the trial included assessment of food effect, evaluation of brain penetration and biomarkers for PPAR Gamma engagement.

The results show that MIN-102 was generally safe and well tolerated at exposures exceeding the levels required for efficacy. No serious adverse events were observed, any adverse events were mild and similar to those observed with the placebo. Results from brain penetration and biomarker assessment confirmed earlier results, showing that MIN-102 reaches its target in the central nervous system and exerts a broad range of effects to treat various aspects of X-ALD aligned with those

observed in preclinical studies. No relevant food effects were observed and pharmacokinetics showed good linearity with dose, providing a simple and convenient dosing regimen for patients.

Based on the successful completion of the phase 1 trial, a phase 2/3 trial in adult AMN patients will be launched in the coming months.

"It is very encouraging to see that MIN-102, as a PPAR gamma agonist, is able to achieve an effect size at the receptor site that cannot be achieved with the approved doses of its parent compound, pioglitazone," said Dr. Uwe Meya, CMO of Minoryx Therapeutics. "We are looking forward to initiating the phase 2/3 trial for MIN-102."

"We are very pleased with the results of the phase 1 study, which provide us with a strong scientific basis to plan and conduct clinical trials in adult patients suffering with AMN," said Dr. Marc Martinell, CEO of Minoryx Therapeutics.